

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
16 June 2005 (16.06.2005)

PCT

(10) International Publication Number
WO 2005/053601 A2

(51) International Patent Classification⁷: **A61K**

(21) International Application Number:
PCT/IL2004/001046

(22) International Filing Date:
15 November 2004 (15.11.2004)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
60/525,883 1 December 2003 (01.12.2003) US

(71) Applicant (for all designated States except US): **TECH-
NION RESEARCH & DEVELOPMENT FOUNDA-
TION LTD.?** [IL/IL]; Senate House, Technion City,
32000 Haifa (IL).

(72) Inventors; and

(75) Inventors/Applicants (for US only): **AMIT, Michal**
[IL/IL]; 261 Yuvalim, 20142 Misgav (IL). **IT-
SKOVITZ-ELDOR, Joseph** [IL/IL]; 42 SheErit HaPleta
Street, 34987 Haifa (IL).

(74) Agent: **G.E. EHRLICH (1995) LTD.**; 11 Menachem Be-
gin Street, 52 521 Ramat Gan (IL).

(81) Designated States (unless otherwise indicated, for every
kind of national protection available): AE, AG, AL, AM,
AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN,
CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI,
GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE,
KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD,
MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG,
PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM,
TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM,
ZW.

(84) Designated States (unless otherwise indicated, for every
kind of regional protection available): ARIPO (BW, GH,
GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM,
ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM),
European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI,
FR, GB, GR, HU, IE, IS, IT, LU, MC, NL, PL, PT, RO, SE,
SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ,
GW, ML, MR, NE, SN, TD, TG).

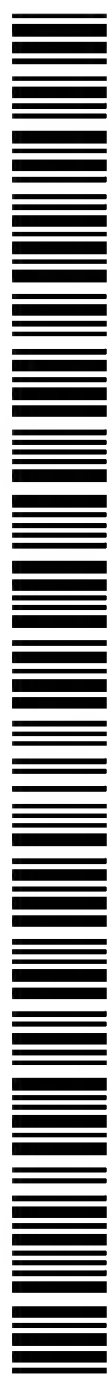
Published:

— without international search report and to be republished
upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guid-
ance Notes on Codes and Abbreviations" appearing at the begin-
ning of each regular issue of the PCT Gazette.

(54) Title: METHODS OF GENERATING STEM CELLS AND EMBRYONIC BODIES CARRYING DISEASE-CAUSING MU-
TATIONS AND METHODS OF USING SAME FOR STUDYING GENETIC DISORDERS

(57) Abstract: Stem cells, stem cell lines and differentiated cells, tissues and organs which carry disease-causing mutations are provided. There is also provided a method of identifying agents suitable for treating disorders associated with at least one disease-causing mutations such as myotonic dystrophy and van Waardenburg syndrome.



WO 2005/053601 A2